
Human retinal progenitor cells as candidate therapy for retinitis pigmentosa

Grant Award Details

Human retinal progenitor cells as candidate therapy for retinitis pigmentosa

Grant Type: Early Translational II

Grant Number: TR2-01794

Project Objective: The objective of the project is to develop a cell-based treatment for the inherited orphan disease retinitis pigmentosa (RP). The strategy is to use intraocular/intervitrius injection of human retinal progenitor cells obtained from fetal eye.

Investigator:

Name: Henry Klassen

Institution: University of California, Irvine

Type: PI

Disease Focus: Vision Loss

Human Stem Cell Use: Adult Stem Cell

Cell Line Generation: Adult Stem Cell

Award Value: \$1,803,768

Status: Closed

Progress Reports

Reporting Period: Year 1

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Grant Application Details

Application Title: Human retinal progenitor cells as candidate therapy for retinitis pigmentosa

Public Abstract:

The targeted disease is retinitis pigmentosa (RP), is a severe form of blindness that runs in families. This disease is not overly common, yet represents an attainable near term target for stem cell therapy for a number of reasons: 1) RP destroys the light detecting cells of the retina but generally leaves the rest of the visual system and body unharmed, so the clinical goal is circumscribed; 2) RP is prototypical of degenerations of the nervous system, so a cure for this less common disease would accelerate progress towards new therapies for a range of more familiar conditions; 3) scientific research has shown that degenerating rods and cones can be spared in animals by transplanting particular types of stem cells, so the scientific feasibility of treating RP in this way has already been established in principle.

The therapeutic approach is to save the light sensing cells of the eye (rod and cone photoreceptors) in people going blind using a type of stem cell obtained from the immature retina, but not from early embryos. These particular stem cells from the retina, known as progenitor cells, are capable of rescuing photoreceptors from degeneration following transplantation to the eye. These same cells are also highly efficient at becoming rod photoreceptors and this provides another more sustained pathway by which they preserve the crucial cone photoreceptors. In addition, there is evidence that the stem cells themselves might become functional photoreceptors and thereby stabilize the retina by directly replacing the dying cells in the patient's eye. Thus, transplanted stem cells could treat the targeted disease of RP in multiple ways simultaneously. Importantly, there are a host of reasons why clinical trials in the eye are easier and safer than most locations in the body. The eye is an important proving ground for stem cell-based therapies and provides a stepping stone to many otherwise incurable diseases of the brain and spinal cord.

Statement of Benefit to California:

Benefits to the state of California and its citizens are both direct and indirect. The direct benefit is medical in that there is currently no cure or established treatment for the individuals and families that suffer from the dreadful hereditary blindness known as retinitis pigmentosa. In addition, there are many people in California and throughout the world that suffer from degenerative diseases of the retina and central nervous system that could benefit from further development and alternate applications of the type of stem cell therapy proposed in the current application. The rapid progress that could be achieved via this proposal would help legitimize the use of stem cells and should thereby accelerate the development of stem cell-based therapeutics for a wide range of other diseases. In so doing there would be an indirect benefit to California by making our state a focal point for stem cell breakthroughs. This would increase medical capabilities, strengthen the [REDACTED], and energize local biotechnology companies with outside investment and a payoff in jobs and tax revenues.

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